

Cardior Pharmaceuticals Demonstrates Reversal of Cardiac Hypertrophy by H19 Gene Therapy

- *Administration of AAV9 expressing H19 attenuates cardiac hypertrophy in vivo and prevents progression to heart failure*
- *Details on novel mechanism of action published in peer-reviewed study in the European Heart Journal*

Hanover, Germany, July 13, 2020 - Cardior Pharmaceuticals GmbH, a clinical-stage biotech company focused on the development of noncoding RNA (ncRNA) therapeutics for patients with cardiovascular diseases, today announced the results of extensive in vitro and in vivo studies demonstrating the therapeutic potential of H19 gene therapy to combat cardiac hypertrophy and heart failure. Results were published in the [European Heart Journal](https://doi.org/10.1093/eurheartj/ehaa519) (doi: 10.1093/eurheartj/ehaa519) [1]. Research was performed by an international team of researchers from Germany, Austria, and the U.S.

H19 is an IP-protected target describing a long noncoding RNA (lncRNA) that is transcribed from a highly species-conserved gene locus. H19 expression is downregulated in failing hearts of mice, pigs and humans. To test the idea that H19 might have cardiomyocyte-specific protective functions and therefore may be used as a therapeutic target, the researchers explored a cardiomyocyte-directed gene therapy using an AAV9 vector delivering the murine or the human H19 gene. Gene delivery was not only safe and well tolerated but expression of the H19 lncRNA also led to significantly attenuated heart failure even when cardiac hypertrophy was already established.

The researchers also identified a link between H19 and pro-hypertrophic nuclear factor of activated T cells (NFAT) signaling, further strengthening the hypothesis that the suppression of this long non-coding RNA plays a crucial role in the development of heart failure.

“We were able to demonstrate that H19 gene therapy prevents and reverses experimental, pressure-overload-induced heart failure,” said Dr. Thomas Thum, Professor at Hannover Medical School, CSO of Cardior and senior author of the study. “H19 acts as an anti-hypertrophic lncRNA and represents a promising therapeutic target to combat pathological cardiac remodeling.”

“H19 is a target of our proprietary RNA portfolio,” said Claudia Ulbrich, CEO of Cardior. “The impressive results of this study once again demonstrate the huge potential of RNA-based medicine for causal therapies of complex diseases. We are delighted to be among the pioneers of this emerging new paradigm.”

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[1] Viereck J et al, Targeting muscle-enriched long non-coding RNA H19 reverses pathological cardiac hypertrophy”, *European Heart Journal*. doi:10.1093/eurheartj/ehaa519

After publication the paper can be found on the European Heart Journal website at:

<https://academic.oup.com/eurheartj/article-lookup/doi/10.1093/eurheartj/ehaa519>

About Cardior

Cardior Pharmaceuticals is a privately held German biopharmaceutical company pioneering the development of curative and preventive heart failure therapeutics based on non-coding RNA (ncRNA). Cardior's therapeutic approach is using distinctive ncRNA signatures driving the molecular reprogramming that causes maladaptive remodeling and heart failure. Drug candidates developed by Cardior represent first-in-class ncRNA therapeutics and diagnostics for patients with myocardial infarction and heart failure. Founded in 2016 based on the work of cardiologist Prof. Dr. Dr. Thomas Thum of Hannover Medical School, the Company has raised EUR 15 Mio. from international investors LSP Life Sciences Partners, BioMedPartners, Boehringer Ingelheim Venture Fund (BIVF), Bristol-Myers Squibb (BMS) and High-Tech Gründerfonds (HTGF).

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